

BioBasics 201: Targeted Biologics for the Non-Scientist

Vaccines, Cell, Gene, Antibody, and RNA Therapies

Level Two | Suggested prerequisite BioBasics 101

BioBasics 201: Targeted Biologics for the Non-Scientist is an intensive, two-day course focused on vaccines, cell therapies, gene therapies, therapeutic antibodies, and RNA-based drugs. Biopharma's inspiration for the development of targeted biologics is our own human immune system, so we begin with an in-depth explanation of immunology. The course then focuses on the science, challenges, and medical promise of next generation targeted biologics. BioBasics 201 is designed for the non-scientist who has taken BioBasics 101 or for those who understand the basics of DNA, RNA, proteins, and cell signaling. Taught by industry experts who explain how these complex drugs work in a simple, engaging manner.

Five Takeaways

1. Understand the mechanisms used by the human body to protect itself against disease.
2. Fluency in vaccine platforms, how each protects against disease including Covid-19.
3. Rational of therapeutic antibody mechanisms of action, including monoclonal antibodies, antibody-drug conjugates, bispecific antibodies, checkpoint inhibitors.
4. Compare and contrast types of DNA- and RNA-based therapies and how each cures disease.
5. Increased knowledge of cell therapies, including all variations of CAR-based therapies.

Agenda

Day One

Immunology: Intro to the Human Immune System *60 minutes*

Tissues of the immune system
Non-specific and specific immunity
Key immune cell roles
Immune signaling: cytokines
Industry application: cytokine storm

Discussion/Break *15 minutes*

Immunology: How Our Body Fights Disease *continued*

Immunology: How Our Body Fights Disease *60 minutes*

Non-specific immune response
Industry application: inflammation
Specific immune response
Activation of the immune system
B-cells
Antibodies: structure and function
Industry application: monoclonal antibodies

Complement response
T-cells
Regulation of the immune system
PD-1 and CTLA-4
Industry application: tumor suppression of T-cells

Discussion/Break 15 minutes

Immunotherapies: An Overview 30 minutes

Immunotherapy defined
Immunotherapies review
Therapeutic antibodies
Oncolytic virus therapy
Vaccines
Cell therapies (CAR-T)

Lunch 45 minutes

Targeted Biologics: Vaccines 45 minutes

Immunological memory
How vaccines work
Vaccine platforms
DNA and RNA vaccines
Industry application: covid vaccine
Industry application: universal flu vaccine

Discussion/Break 15 minutes

Focus On: Oncology 45 minutes

Cancer
Growth factor signaling
Industry application: Gleevec
Immunosuppressive tumor microenvironment
Cancer immunotherapy

Wrap-Up 15 minutes

Day Two

Targeted Biologics: Therapeutic Antibodies
60 minutes

Therapeutic antibodies
Industry application: polyclonal vs monoclonal antibodies
Therapeutic antibody mechanisms of action
Antibody-drug conjugates

Targeted Biologics: Gene Therapies *continued*
Industry application: Zolgensma

Targeted Biologics: Therapeutic Antibodies
continued

Bispecific antibodies
Checkpoint inhibitors
Industry application: PD-1 and PD-L1
Industry application: CTLA-4
Next generation checkpoint inhibitors

Discussion/Break 15 minutes

Targeted Biologics: Cell Therapies 60 minutes

How immune cells are used for cell therapy
CAR structure and function
Selected CAR therapies
CAR variations: CAR-NK, CAR-MA TCR
Industry application: targeting solid tumors
Autologous vs allogeneic cell therapies
How are CARs made?
CAR-T safety: controlling activation
Industry application: CAR treatment for autoimmunity

Discussion/Break 15 minutes

Targeted Biologics: RNA Therapies
60 minutes

RNA's role in the cell
RNA's role in disease
Therapeutic areas
Types of RNA-based therapeutics
Antisense
Industry application: Kynamro
Exon-inclusion and exon-skipping
Industry application: Spinraza
siRNA therapies

Lunch 45 minutes

Targeted Biologics: Gene Therapies 45 minutes

Gene therapy: in vivo and ex vivo
DNA deliver via viral vectors
Viral vector platforms
Gene therapy composition
AAV and lentivirus characteristics
Industry application: Luxturna

AAV neutralizing antibodies
Gene therapy and biomarkers
Durability of effect
RMAT designation
Risks and challenges

Targeted Biologics: Genome Editing *60 minutes*

Gene therapy vs genome editing

Zinc finger nucleases (ZFN)

 ZFN therapeutic areas

 How ZFN work

 ZFN in the clinic

 ZFN Safety

CRISPR

 CRISPR therapeutic areas

 How CRISPR works

 CRISPR Safety

 CRISPR in the clinic

 Industry application: PD-1 knockouts

 CRISPR Babies activity

 CRISPR as RNA editor

 CRISPR diagnostics

 Industry application: SHERLOCK and
 DETECTR

Wrap-Up *15 minutes*